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APPLICATION NO.	FILING DATE	FIRST NAMED INVENTOR	ATTORNEY DOCKET NO.	CONFIRMATION NO.
09/763,535	02/20/2001	Guy Rousseau	VANM198.001A	7462

7590

12/17/2003

Knobbe Martens Olsons & Bear  
620 Newport Center Drive Sixteenth Floor  
Newport Beach, CA 92660

EXAMINER
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SULLIVAN, DANIEL M

ART UNIT	PAPER NUMBER
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1636

DATE MAILED: 12/17/2003

Please find below and/or attached an Office communication concerning this application or proceeding.

## Office Action Summary

Application No.

09/763,535

Applicant(s)

ROUSSEAU ET AL.

Examiner

Daniel M Sullivan

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-- The MAILING DATE of this communication appears on the cover sheet with the correspondence address --

### Period for Reply

A SHORTENED STATUTORY PERIOD FOR REPLY IS SET TO EXPIRE 3 MONTH(S) FROM THE MAILING DATE OF THIS COMMUNICATION.

- Extensions of time may be available under the provisions of 37 CFR 1.136(a). In no event, however, may a reply be timely filed after SIX (6) MONTHS from the mailing date of this communication.
- If the period for reply specified above is less than thirty (30) days, a reply within the statutory minimum of thirty (30) days will be considered timely.
- If NO period for reply is specified above, the maximum statutory period will apply and will expire SIX (6) MONTHS from the mailing date of this communication.
- Failure to reply within the set or extended period for reply will, by statute, cause the application to become ABANDONED (35 U.S.C. § 133).
- Any reply received by the Office later than three months after the mailing date of this communication, even if timely filed, may reduce any earned patent term adjustment. See 37 CFR 1.704(b).

### Status

- 1) ☒ Responsive to communication(s) filed on 25 September 2003.
- 2a) ☒ This action is **FINAL**. 2b) ☐ This action is non-final.
- 3) ☐ Since this application is in condition for allowance except for formal matters, prosecution as to the merits is closed in accordance with the practice under *Ex parte Quayle*, 1935 C.D. 11, 453 O.G. 213.

### Disposition of Claims

- 4) ☒ Claim(s) 1 and 4-9 is/are pending in the application.
- 4a) Of the above claim(s) \_\_\_\_\_ is/are withdrawn from consideration.
- 5) ☐ Claim(s) \_\_\_\_\_ is/are allowed.
- 6) ☒ Claim(s) 1 and 4-9 is/are rejected.
- 7) ☐ Claim(s) \_\_\_\_\_ is/are objected to.
- 8) ☐ Claim(s) \_\_\_\_\_ are subject to restriction and/or election requirement.

### Application Papers

- 9) ☐ The specification is objected to by the Examiner.
- 10) ☒ The drawing(s) filed on 25 September 2003 is/are: a) ☒ accepted or b) ☐ objected to by the Examiner.  
Applicant may not request that any objection to the drawing(s) be held in abeyance. See 37 CFR 1.85(a).  
Replacement drawing sheet(s) including the correction is required if the drawing(s) is objected to. See 37 CFR 1.121(d).
- 11) ☐ The oath or declaration is objected to by the Examiner. Note the attached Office Action or form PTO-152.

### Priority under 35 U.S.C. §§ 119 and 120

- 12) ☐ Acknowledgment is made of a claim for foreign priority under 35 U.S.C. § 119(a)-(d) or (f).  
a) ☐ All b) ☐ Some \* c) ☐ None of:  
1. ☐ Certified copies of the priority documents have been received.  
2. ☐ Certified copies of the priority documents have been received in Application No. \_\_\_\_\_.  
3. ☐ Copies of the certified copies of the priority documents have been received in this National Stage application from the International Bureau (PCT Rule 17.2(a)).  
\* See the attached detailed Office action for a list of the certified copies not received.
- 13) ☐ Acknowledgment is made of a claim for domestic priority under 35 U.S.C. § 119(e) (to a provisional application) since a specific reference was included in the first sentence of the specification or in an Application Data Sheet. 37 CFR 1.78.  
a) ☐ The translation of the foreign language provisional application has been received.
- 14) ☐ Acknowledgment is made of a claim for domestic priority under 35 U.S.C. §§ 120 and/or 121 since a specific reference was included in the first sentence of the specification or in an Application Data Sheet. 37 CFR 1.78.

### Attachment(s)

- 1) ☐ Notice of References Cited (PTO-892) 4) ☐ Interview Summary (PTO-413) Paper No(s) \_\_\_\_\_
- 2) ☐ Notice of Draftsperson's Patent Drawing Review (PTO-948) 5) ☐ Notice of Informal Patent Application (PTO-152)
- 3) ☐ Information Disclosure Statement(s) (PTO-1449) Paper No(s) \_\_\_\_\_ 6) ☐ Other: \_\_\_\_\_

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### **DETAILED ACTION**

This Office Action is a reply to the "AMENDMENT" of 25 September 2003 (hereinafter, 25 September Paper) filed in response to the Non-Final Office Action mailed 19 June 2003 (hereinafter, 19 June Office Action). Claims 1 and 4-9 were considered in the 19 June Office Action. Claim 8 was amended in the 25 September Paper. Claims 1 and 4-9 are presently pending and under consideration.

### ***Response to Amendment***

#### Drawings

The formal drawings submitted with the 25 September Paper are approved.

#### Claim Objections

Objection to claim 8 is withdrawn.

#### Claim Rejections - 35 USC § 112

Claims 1 and 4-9 stand rejected under 35 U.S.C. §112, first paragraph, as lacking enablement for reasons of record in Paper No. 8 and herein below in the "Response to Arguments".

Rejection of claim 8 under 35 U.S.C. 112, second paragraph, as indefinite is withdrawn.

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Claim Rejections - 35 USC § 102

Rejection of claims 1, 3 and 5 under 35 U.S.C. 102(a) as anticipated by Jacquemin *et al.* (1999) *J. Biol. Chem.* 274:2665-2671 is withdrawn in view of the perfection of the priority claim under 35 U.S.C. §119.

***Response to Arguments***

Claim Rejections - 35 USC § 112

Claims 1 and 4-9 were rejected under 35 U.S.C. §112, first paragraph, as lacking enablement for pharmaceutical compositions comprising one cut polypeptides, nucleic acids encoding said one cut polypeptides or cells expressing said one cut polypeptides, or for therapeutic methods comprising administering the claimed pharmaceutical compositions.

In response to the rejection and arguments of record, Applicant submits two publications alleged to provide further enabling teachings with respect to the transfection of human and animal cells with an expression vector comprising the claimed polynucleotides. Applicant identifies *Biochem. Biophys. Res. Commun.* 292, 848-854 (2002) as demonstrating transfection of COS-7 and HEK-293 cells with an OC-3 expression vector and correct translation of the gene in said cells, as well as apparent OC-3-responsive promoter activation upon transfection of HEK-293 cells. Applicant further submits *Biochem. Biophys. Res. Commun.* 285, 1200-1205 (2001) wherein transcription factor OC-2 controls the Microphthalmia-associated transcription factor gene.

The Attachments have been fully considered but are not found persuasive. As has been pointed out in previous Office Actions, the grounds set forth for the enablement rejection are,

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“the art recognized unpredictability of achieving therapeutic levels of gene expression following direct or indirect administration of nucleic acid vectors, and the unpredictability of extending the results of animal systems to humans, the lack of guidance provided by the specification for the parameters affecting delivery and expression of therapeutic amounts of DNA into the cells, the lack of guidance concerning the treatment of diabetes, cancer and Waardenburg syndrome using the polynucleotide of the instant invention” (Paper No. 8, page 7). The exhibits demonstrate expression of OC-2 and OC-3 in COS-7 and HEK-293 cells *in vitro*. Neither of these systems is recognized in the art as a model for any disease, and obtaining heterologous expression of a protein in COS-7 or HEK-293 cells is not recognized as enabling for a method of gene therapy. Therefore, the skilled artisan would not know how to treat diabetes, cancer, Waardenburg syndrome or any other disease based on the teachings of the Exhibits.

Applicant further argues that clinical data is not required to meet the enablement requirement of 35 U.S.C. §112, first paragraph and reiterates a discussion of the teachings of the specification that was addressed in the previous Office Action. The claims are rejected based on teachings from the relevant art which indicate that obtaining a therapeutic effect using gene therapy techniques available in the art at the time of filing was highly unpredictable. The teachings from the specification cited by Applicant in the response are merely general statements that the pharmaceutical compositions will be used to treat various conditions and non-specific descriptions of methods of administering pharmaceutical compositions which are well known to the skilled artisan. While it is acknowledged that the specification need not contain a working example if the invention is otherwise disclosed in such manner that one skilled in the art will be able to practice it without an undue amount of experimentation, lack of a working example is a

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factor to be considered, especially in a case involving an unpredictable and undeveloped art. The previous Office Actions clearly establish the nature of the art of gene therapy for any condition as unpredictable. Thus, given no more than the general teachings and prophetic statements of the specification and the tissue culture experiments of the cited art, the skilled artisan would not know how to use a pharmaceutical composition comprising a polynucleotide encoding OC-2, a vector comprising said polynucleotide, an OC-2 protein or a cell line transformed with said vector to treat any condition. Nor would the skilled artisan know how to treat cancer diabetes or Waardenburg syndrome according to the claims. For these reasons and reasons previously made of record the claims stand rejected as lacking an enabling disclosure.

### ***Conclusion***

**THIS ACTION IS MADE FINAL.** Applicant is reminded of the extension of time policy as set forth in 37 CFR 1.136(a).

A shortened statutory period for reply to this final action is set to expire THREE MONTHS from the mailing date of this action. In the event a first reply is filed within TWO MONTHS of the mailing date of this final action and the advisory action is not mailed until after the end of the THREE-MONTH shortened statutory period, then the shortened statutory period will expire on the date the advisory action is mailed, and any extension fee pursuant to 37 CFR 1.136(a) will be calculated from the mailing date of the advisory action. In no event, however, will the statutory period for reply expire later than SIX MONTHS from the mailing date of this final action.


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Any inquiry concerning this communication or earlier communications from the examiner should be directed to Daniel M Sullivan whose telephone number is 703-305-4448. The examiner can normally be reached on Monday through Friday 8-4:30.

If attempts to reach the examiner by telephone are unsuccessful, the examiner's supervisor, Remy Yucel, Ph.D. can be reached on 703-305-1998. The fax phone number for the organization where this application or proceeding is assigned is 703-872-9306.

Any inquiry of a general nature or relating to the status of this application or proceeding should be directed to the receptionist whose telephone number is 703-308-0196.

DMS

  
DAVID GUZO  
PRIMARY EXAMINER